

(19) World Intellectual Property
Organization
International Bureau



(43) International Publication Date
19 February 2004 (19.02.2004)

PCT

(10) International Publication Number
WO 2004/015075 A3

(51) International Patent Classification⁷: C12Q 1/68,
C07H 21/02

(21) International Application Number:
PCT/US2003/024658

(22) International Filing Date: 5 August 2003 (05.08.2003)

(25) Filing Language: English

(26) Publication Language: English

(30) Priority Data:
60/401,943 8 August 2002 (08.08.2002) US

(71) Applicant: DHARMACON, INC. [US/US]; 1376 Miners
Drive #101, Lafayette, CO 80026 (US).

(72) Inventor: SCARINGE, Stephen; 2746 Prairie Ridge,
lafayette, CO 80026 (US).

(74) Agents: KALOW, David, A. et al.; Kalow & Springut
LLP, 19th floor, 488 Madison Avenue, New York, NY
10022 (US).

(81) Designated States (national): AE, AG, AL, AM, AT, AU,
AZ, BA, BB, BG, BR, BY, BZ, CA, CH, CN, CO, CR, CU,

CZ, DE, DK, DM, DZ, EC, EE, ES, FI, GB, GD, GE, GH,
GM, HR, HU, ID, IL, IN, IS, JP, KE, KG, KP, KR, KZ, LC,
LK, LR, LS, LT, LU, LV, MA, MD, MG, MK, MN, MW,
MX, MZ, NI, NO, NZ, OM, PG, PH, PL, PT, RO, RU, SC,
SD, SE, SG, SK, SL, SY, TJ, TM, TN, TR, TT, TZ, UA,
UG, UZ, VC, VN, YU, ZA, ZM, ZW.

(84) Designated States (regional): ARIPO patent (GH, GM,
KE, LS, MW, MZ, SD, SL, SZ, TZ, UG, ZM, ZW),
Eurasian patent (AM, AZ, BY, KG, KZ, MD, RU, TJ, TM),
European patent (AT, BE, BG, CH, CY, CZ, DE, DK, EE,
ES, FI, FR, GB, GR, HU, IE, IT, LU, MC, NL, PT, RO,
SE, SI, SK, TR), OAPI patent (BF, BJ, CF, CG, CI, CM,
GA, GN, GQ, GW, ML, MR, NE, SN, TD, TG).

Published:

- with international search report
- before the expiration of the time limit for amending the
claims and to be republished in the event of receipt of
amendments

(88) Date of publication of the international search report:
17 June 2004

*For two-letter codes and other abbreviations, refer to the "Guid-
ance Notes on Codes and Abbreviations" appearing at the begin-
ning of each regular issue of the PCT Gazette.*

WO 2004/015075 A3

(54) Title: SHORT INTERFERING RNAs HAVING A HAIRPIN STRUCTURE CONTAINING A NON-NUCLEOTIDE LOOP

(57) Abstract: This invention provides a short interfering hairpin RNA having the structure X_1 -L- X_2 , wherein X_1 and X_2 are nucleotide sequences having sufficient complementarity to one another to form a double-stranded stem hybrid and L is a loop region comprising a non-nucleotide linker molecule, wherein at least a portion of one of the nucleotide sequences located within the double-stranded stem is complementary to a sequence of said target RNA.